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ALGLUCERASE CASE STUDY ILLUSTRATES CHALLENGES OF CONTROLLING HEALTH CARE COSTS

A new drug treatment could cost at least \$71,000 and as much as a half million dollars per year per patient, says the congressional Office of Technology Assessment (OTA).

Alglucerase is a new therapy for the uncommon inherited disorder called Gaucher disease. The disease is caused by deficient activity of an enzyme, leading to significant disability and sometimes death. An estimated 2,100 to 11,000 U.S. residents are believed to have symptoms severe enough to warrant medical care.

In a background paper released today, OTA analyzes the the development of alglucerase as a case study dramatically illustrating the challenges Americans face in devising policies to control health care costs without deterring the development of effective new technologies.

Alglucerase, marketed under the brand name CeredaseTM by Genzyme, Inc., a Massachusetts pharmaceutical firm, is a chemical derivative of the deficient enzyme that causes Gaucher disease. Prior to its development, there was no effective treatment for the disease.

The drug was developed through significant investment by both the federal government, particularly the National Institutes of Health (NIH), and the private sector. Its high cost to consumers, private insurers, and the federal government raises questions about the extent to which NIH is acting in the public interest in providing significant assistance to some medical technologies ultimately marketed by the private sector. The actions of NIH, which sponsored or performed most of the scientific research that led to the discovery of alglucerase, can have consequences far beyond providing new therapies to combat disease, says OTA.

As the case illustrates, such deep involvement by NIH creates the potential for the federal government (i.e. the public) to pay for such technologies twice -- once through support of the R&D process, and once again as a health insurer. The federal government has no mechanism to ensure that the prices Americans pay for drugs and other technologies reflect the public's contribution to their development, says OTA.

OTA points out that the Orphan Drug Act and NIH's involvement together significantly reduced the cost and removed much of the risk that pharmaceutical companies customarily face when they decide to develop a drug. When alglucerase was officially designated an orphan drug, Genzyme was entitled to 7 years of exclusive marketing, greatly enhancing the drug's potential profitability.

On the basis of information supplied by Genzyme, OTA estimates that the firm spent approximately \$29.4 million on R&D for alglucerase over the decade prior to its approval for marketing in 1991. Although Genzyme claims that it spent about \$48.6 million in cash outlays, OTA includes only actual expenditures for the work, materials, and facilities needed for the conduct of R&D.

Revenues to Genzyme from patients and their insurers under the Food and Drug Administration's (FDA) Treatment Investigational New Drug Program (IND) exceeded \$5 million. This program provides patients not enrolled in clinical trials with access to experimental drugs for otherwise untreatable conditions. The FDA allowed Genzyme to charge a price to recover costs associated with the R&D and provision of the drug.

Because the vast majority of private health insurance policies impose a limit on total benefits payable for each insuree, somewhere between one-third and one-half of all alglucerase recipients face a significant risk of exhausting or critically reducing their available insurance benefits over time. Although Genzyme supplies the drug free to those patients who exhaust (or otherwise lack) insurance benefits, OTA

points out that for the rest of their lives such patients remain uninsured for the cost of administering alglucerase and any other medical expenses they may incur.

OTA concludes that perhaps the most significant lesson to emerge from the case of alglucerase is that cost considerations cannot be ignored in the development and diffusion of any treatment. Payments for alglucerase, like expenditures for any other treatment or diagnostic procedure, divert health care resources from other uses.

Copies of the 34-page background paper *Federal and Private Roles in the Development and provision of Alglucerase Therapy for Gaucher Disease* for congressional use are available by calling 4-9241. Copies for non-congressional use can be ordered from the Superintendent of Documents, U.S. Government Printing Office (GPO), Washington, D.C. 20402-9325; phone (202) 783-3238. The GPO stock number is 052-003-01309-6; the price is \$2.75.

OTA is a nonpartisan analytical agency that serves the U.S. Congress. Its purpose is to aid Congress in dealing with the complex and often highly technical issues that increasingly confront our society.

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